



# SEQUENCE LISTING

<110> Hildinger, Markus

<120> Decreasing gene expression in a mammalian subject in vivo via  
AAV-mediated RNAi expression cassette transfer

<130> 1339

<140> US 10/604,340

<141> 2003-07-13

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<170> PatentIn version 3.1

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<223> sequence for recombinant adeno-associated viral vector, including  
plasmid backbone, with AAV2 internal terminal repeats that flank  
expression cassette; referred to as AAV2/2 CMV luciferase

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 expression cassette; referred to as AAV2/2 CMV luciferase  
  
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<223> sequence for recombinant adeno-associated viral vector, including  
plasmid backbone, with AAV2 internal terminal repeats that flank  
expression cassette; referred to as AAV2/5 CMV luciferase

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Gly Pro Ala Pro Phe Tyr Pro Leu Glu Asp Gly Thr Ala Gly Glu Gln	
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Leu His Lys Ala Met Lys Arg Tyr Ala Leu Val Pro Gly Thr Ile Ala	
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Phe Thr Asp Ala His Ile Glu Val Asp Ile Thr Tyr Ala Glu Tyr Phe	
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aac gac att tat aat gaa cgt gaa ttg ctc aac agt atg ggc att tcg Asn Asp Ile Tyr Asn Glu Arg Glu Leu Leu Asn Ser Met Gly Ile Ser 110 115 120	1590
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gat act gcg att tta agt gtt gtt cca ttc cat cac ggt ttt gga atg Asp Thr Ala Ile Leu Ser Val Val Pro Phe His His Gly Phe Gly Met 235 240 245	1974
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tat aga ttt gaa gaa gag ctg ttt ctg agg agc ctt cag gat tac aag Tyr Arg Phe Glu Glu Glu Leu Phe Leu Arg Ser Leu Gln Asp Tyr Lys 270 275 280	2070

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 <213> Artificial

<220>  
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 plasmid backbone, with AAV2 internal terminal repeats that flank  
 expression cassette; referred to as AAV2/5 CMV luciferase

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Leu Glu Asp Gly Thr Ala Gly Glu Gln Leu His Lys Ala Met Lys Arg  
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Tyr Ala Leu Val Pro Gly Thr Ile Ala Phe Thr Asp Ala His Ile Glu  
 35 40 45

Val Asp Ile Thr Tyr Ala Glu Tyr Phe Glu Met Ser Val Arg Leu Ala  
 50 55 60

Glu Ala Met Lys Arg Tyr Gly Leu Asn Thr Asn His Arg Ile Val Val  
 65 70 75 80

Cys Ser Glu Asn Ser Leu Gln Phe Phe Met Pro Val Leu Gly Ala Leu  
 85 90 95

Phe Ile Gly Val Ala Val Ala Pro Ala Asn Asp Ile Tyr Asn Glu Arg  
 100 105 110



Glu Leu Leu Asn Ser Met Gly Ile Ser Gln Pro Thr Val Val Phe Val  
115 120 125

Ser Lys Lys Gly Leu Gln Lys Ile Leu Asn Val Gln Lys Lys Leu Pro  
130 135 140

Ile Ile Gln Lys Ile Ile Ile Met Asp Ser Lys Thr Asp Tyr Gln Gly  
145 150 155 160

Phe Gln Ser Met Tyr Thr Phe Val Thr Ser His Leu Pro Pro Gly Phe  
165 170 175

Asn Glu Tyr Asp Phe Val Pro Glu Ser Phe Asp Arg Asp Lys Thr Ile  
180 185 190

Ala Leu Ile Met Asn Ser Ser Gly Ser Thr Gly Leu Pro Lys Gly Val  
195 200 205

Ala Leu Pro His Arg Thr Ala Cys Val Arg Phe Ser His Ala Arg Asp  
210 215 220

Pro Ile Phe Gly Asn Gln Ile Ile Pro Asp Thr Ala Ile Leu Ser Val  
225 230 235 240

Val Pro Phe His His Gly Phe Gly Met Phe Thr Thr Leu Gly Tyr Leu  
245 250 255

Ile Cys Gly Phe Arg Val Val Leu Met Tyr Arg Phe Glu Glu Glu Leu  
260 265 270

Phe Leu Arg Ser Leu Gln Asp Tyr Lys Ile Gln Ser Ala Leu Leu Val  
275 280 285

Pro Thr Leu Phe Ser Phe Phe Ala Lys Ser Thr Leu Ile Asp Lys Tyr  
290 295 300

Asp Leu Ser Asn Leu His Glu Ile Ala Ser Gly Gly Ala Pro Leu Ser  
305 310 315 320

Lys Glu Val Gly Glu Ala Val Ala Lys Arg Phe His Leu Pro Gly Ile  
325 330 335



Arg Gln Gly Tyr Gly Leu Thr Glu Thr Thr Ser Ala Ile Leu Ile Thr  
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Pro Glu Gly Asp Asp Lys Pro Gly Ala Val Gly Lys Val Val Pro Phe  
 355 360 365

Phe Glu Ala Lys Val Val Asp Leu Asp Thr Gly Lys Thr Leu Gly Val  
 370 375 380

Asn Gln Arg Gly Glu Leu Cys Val Arg Gly Pro Met Ile Met Ser Gly  
 385 390 395 400

Tyr Val Asn Asn Pro Glu Ala Thr Asn Ala Leu Ile Asp Lys Asp Gly  
 405 410 415

Trp Leu His Ser Gly Asp Ile Ala Tyr Trp Asp Glu Asp Glu His Phe  
 420 425 430

Phe Ile Val Asp Arg Leu Lys Ser Leu Ile Lys Tyr Lys Gly Tyr Gln  
 435 440 445

Val Ala Pro Ala Glu Leu Glu Ser Ile Leu Leu Gln His Pro Asn Ile  
 450 455 460

Phe Asp Ala Gly Val Ala Gly Leu Pro Asp Asp Asp Ala Gly Glu Leu  
 465 470 475 480

Pro Ala Ala Val Val Val Leu Glu His Gly Lys Thr Met Thr Glu Lys  
 485 490 495

Glu Ile Val Asp Tyr Val Ala Ser Gln Val Thr Thr Ala Lys Lys Leu  
 500 505 510

Arg Gly Gly Val Val Phe Val Asp Glu Val Pro Lys Gly Leu Thr Gly  
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Lys Leu Asp Ala Arg Lys Ile Arg Glu Ile Leu Ile Lys Ala Lys Lys  
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Gly Gly Lys Ile Ala Val  
 545 550

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 <212> DNA  
 <213> Artificial

<220>  
 <223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 U6 lucRI-1b

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 <212> DNA  
 <213> Artificial

<220>  
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plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 U6/U6 lucRIU6-3

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<220>  
 <223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 U6 lucRI-4(sense)

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<220>  
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 plasmid backbone, with AAV2 internal terminal repeats that flank  
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<223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/2 U6 eGFPRI-1a

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